STATE OF WEST VIRGINIA
DEPARTMENT OF HEALTH AND HUMAN RESOURCES
BUREAU FOR MEDICAL SERVICES

Office of Pharmacy Service
Prior Authorization Criteria

EXONDYS 51® (eteplirsen)

Effective 06/01/2018

Prior Authorization Request Form

EXONDYS 51 is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. This indication is approved under accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with EXONDYS 51. **A clinical benefit of EXONDYS 51 has not been established.** Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

Prior authorization requests for Exondys 51 must be submitted as a medical claim and require review by the Medical Director and are will only be considered if the following criteria are met:

1. Patient must have a confirmed mutation of a DMD gene that is amenable to exon 51 skipping (chart notes required); **AND**

2. Patient must be currently taking a corticosteroid OR have a contraindication to corticosteroids; **AND**

3. Prior authorization requests must be accompanied with peer-reviewed literature **confirming** clinical benefit of this medication in patients diagnosed with DMD. Preliminary trial data may be submitted for consideration but does not assure approval.; **AND**

4. Appropriate and validated baseline function test results must be submitted with the initial request for therapy. These tests may include any of the following:

    a. Ambulatory patients: Six-minute walk test (6MWDT) of > 180 meters.
    b. Non-ambulatory patients: Brooke Upper Extremity Function Scale (of 5 or less) AND a Forced Vital Capacity of ≥ 30% of predicted value.

Prior authorization approvals will be for 6 months. Continuation requests must provide clinical documentation of efficacy as evidenced by improvement or stabilization of functions compared to baseline measures.

References

1.) Exondys 51 Package Insert (Sarepta Therapeutics) – Revised 2/2018
2.) Lexicomp monograph for Exondys 51 – reviewed 5/10/2018
3.) Birnrant et al. Lancet Neurol. 2018 March; 17(3): 251-267. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management
4.) Mendell, JR et al. Ann Neurol 2016;79:257–271. Longitudinal Effect of Eteplirsen versus Historical Control on Ambulation in Duchenne Muscular Dystrophy
5.) Kinane, TB et al. Journal of Neuromuscular Diseases 5 (2018) 47–58 Long-Term Pulmonary Functionin Duchenne Muscular Dystrophy: Comparison of Eteplirsen-Treated Patients to Natural History
6.) Clinical Trials:
    a. https://clinicaltrials.gov/ct2/show/NCT01396239?term=eteplirsen&rank=6
    b. https://clinicaltrials.gov/ct2/show/NCT01540409